Verastem Oncology Reports Third Quarter 2024 Financial Results and Highlights Recent Business Updates

November 6, 2024 at 4:05 PM EST

Completed rolling NDA submission for avutometinib and defactinib combination in recurrent KRAS mutant low-grade serous ovarian cancer in October 2024

Company seeking accelerated approval and priority review of its NDA submission for patients with KRAS mutant low-grade serous ovarian cancer; FDA filing decision expected before the end of 2024 with potential for FDA approval decision by mid-2025

Preparations for a potential U.S. commercial launch in mid-2025 are ongoing

Presented positive, updated safety and efficacy results from the RAMP 201 trial at the IGCS 2024 Annual Meeting in October 2024

BOSTON--(BUSINESS WIRE)--Nov. 6, 2024-- Verastem Oncology (Nasdaq: VSTM), a biopharmaceutical company committed to advancing new medicines for patients with cancer, today announced business updates and reported financial results for the third quarter ended September 30, 2024.

“In the third quarter of 2024, we made advancements in our recurrent low-grade serous ovarian cancer program, including sharing updated Phase 2 RAMP 201 data demonstrating robust and durable response rates, tumor reductions across a majority of patients regardless of their KRAS mutation status, and low discontinuation rates due to adverse events. We also completed our rolling NDA submission for recurrent KRAS mutant low-grade serous ovarian cancer and strengthened our balance sheet,” said Dan Paterson, president and chief executive officer of Verastem Oncology. “Looking ahead to the fourth quarter of 2024, we anticipate an FDA decision on the acceptance of our NDA, plan to submit our updated RAMP 201 trial data for publication and expect to prepare a U.S. IND application for VS-7375, an oral KRAS G12D (ON/OFF) inhibitor.”

Third Quarter 2024 and Recent Updates

Avutometinib and Defactinib Combination in Low-Grade Serous Ovarian Cancer (LGSOC)

- Completed the rolling New Drug Application (NDA) submission for the combination of avutometinib and defactinib for adult patients with recurrent KRAS mutant LGSOC, who received at least one prior systemic therapy, in October 2024. The Company submitted the NDA under the U.S. Food and Drug Administration (FDA) Accelerated Approval pathway and requested Priority Review based on the combination’s potential to address significant unmet medical need among patients with recurrent LGSOC.
- RAMP 301, which is currently enrolling patients with recurrent LGSOC regardless of KRAS mutation status across the U.S., UK, EU, Canada, and Australia, will serve as a confirmatory study for the initial indication and has potential to expand the indication regardless of KRAS mutation status. The Company plans to complete enrollment in RAMP 301 by the end of 2025. The Company plans to map out a path forward with the FDA for the KRAS wild-type indication, including the ability to leverage data from the ongoing RAMP 301 Phase 3 trial.
- Announced mature data from the RAMP 201 trial that continued to show robust and durable response rates with low discontinuation rates due to adverse events in patients with recurrent KRAS mutant or KRAS wild-type LGSOC who had a minimum follow-up of 12 months, at the International Gynecologic Cancer Society (IGCS) 2024 Annual Meeting on October 17, 2024. The primary analysis of the RAMP 201 trial, with a data cutoff of June 30, 2024, showed a confirmed overall response rate (ORR) by blinded independent central review (BICR) of 31% (34/109; 95% CI: 23-41), 44% (25/57; 95% CI: 31-58) in KRAS mutant LGSOC, and 17% (9/52; 95% CI: 8-30) in KRAS wild-type LGSOC. The majority (82%) of all patients had a reduction in their tumors, regardless of KRAS status. The updated data continue to demonstrate avutometinib in combination with defactinib is generally well-tolerated, with a 10% discontinuation rate due to adverse events (AEs) and no new safety signals.
- The Company continued its commercial preparation activities for a potential U.S. launch in mid-2025.
- The Japanese Gynecologic Oncology Group (JGOG) dosed the first patient in a Phase 2 Verastem sponsored clinical trial, called RAMP201J, evaluating the safety and efficacy of avutometinib in combination with defactinib for recurrent LGSOC in Japan in October 2024.

Avutometinib in Combination with KRAS G12C Inhibitors in Non-Small Cell Lung Cancer (NSCLC)

Following a thorough evaluation of the Company’s lung cancer clinical development program, Verastem has decided to discontinue the Phase 1/2 RAMP 204 clinical trial evaluating the combination of avutometinib and adagrasib in patients with KRAS G12C-mutant NSCLC. There are no safety concerns with the RAMP 204 trial. The Company is prioritizing the Phase 1/2 RAMP 203 clinical trial, which is evaluating the doublet of avutometinib and sotorasib and the triplet combination of avutometinib and sotorasib plus defactinib in similar patient populations.

- Since the last update of RAMP 203 in October of 2023, enrollment to the doublet of avutometinib plus sotorasib for the

KRAS G12C inhibitor naïve Stage I Part B cohort has recently completed, and per protocol patients are being followed to determine if the efficacy supports further expanded enrollment into Stage II. The KRAS G12C inhibitor prior-treated Stage I Part B cohort enrollment is nearly complete.
- Earlier this year, RAMP 203 was modified to include the triplet combination of avutometinib and sotorasib plus defactinib and the first safety cohort of this triplet has been fully enrolled. Preclinical data provide strong evidence that addition of a FAK inhibitor to the sotorasib and avutometinib doublet has the potential to deepen anti-tumor response and significantly delay tumor progression.
- Expect to report updated interim data from the doublet combination of avutometinib plus sotorasib and provide initial safety data and a status of enrollment for the triplet combination of avutometinib, sotorasib and defactinib in the RAMP 203 trial by the end of 2024.

Avutometinib and Defactinib Combination in First-Line Metastatic Pancreatic Cancer

- Preclinical data outlining the scientific rationale for the combination of avutometinib plus defactinib with standard of care chemotherapy was published in the October 23, 2024 edition of *Science Translational Medicine*.
- Presented initial interim safety and efficacy results from the ongoing RAMP 205 trial of avutometinib and defactinib in combination with current standard of care gemcitabine and nab-paclitaxel in first-line metastatic pancreatic cancer on June 1, 2024, at the American Society of Clinical Oncology (ASCO) Annual Meeting.
- Expect to report updated data from the ongoing RAMP 205 trial in Q1 2025.

VS-7375/GFH375: Oral KRAS G12D (ON/OFF) Inhibitor

- GenFleet began dosing several patients in the Phase 1/2 trial in China evaluating VS-7375/GFH375 in patients with KRAS G12D-mutated advanced solid tumors in July 2024.
- After evaluating initial dose escalation data from the Phase 1 study of VS-7375/GFH375 in China, Verastem anticipates filing a U.S. investigational new drug (IND) application by Q1 2025.
- Discovery/lead optimization continues for the second and third programs in the GenFleet collaboration.

Third Quarter 2024 Financial Results

Verastem Oncology ended the third quarter of 2024 with cash, cash equivalents and short-term investments of $113.2 million which provides an expected cash runway through the potential approval of avutometinib and defactinib for recurrent LGSOC in mid-2025.

Total operating expenses for the three months ended September 30, 2024 (the “2024 Quarter”) were $37.0 million, compared to $21.3 million for the three months ended September 30, 2023 (the “2023 Quarter”).

Research & development expenses for the 2024 Quarter were $24.8 million, compared to $13.9 million for the 2023 Quarter. The increase of $10.9 million, or 78.4%, was primarily related to contract research organization costs, consulting costs, investigator fees associated with ensuring continued rapid start-up of RAMP 301, and a clinical milestone expense that was reached in the GenFleet G12D program.

Selling, general & administrative expenses for the 2024 Quarter were $12.3 million, compared to $7.4 million for the 2023 Quarter. The increase of $4.9 million, or 66.2%, was primarily related to a one-time cost associated with July 2024 financing activities, personnel costs, including non-cash stock compensation and additional costs in anticipation of a potential launch of avutometinib and defactinib in LGSOC.

Net loss for the 2024 Quarter was $24.0 million, or $0.60 per share (basic and diluted), compared to $20.0 million, or $0.75 per share (basic and diluted) for the 2023 Quarter.

For the 2024 Quarter, non-GAAP adjusted net loss was $35.3 million, or $0.88 per share (diluted) compared to non-GAAP adjusted net loss of $19.0 million, or $0.71 per share (diluted) for the 2023 Quarter. Please refer to the GAAP to non-GAAP Reconciliation attached to this press release.

Use of Non-GAAP Financial Measures

To supplement Verastem Oncology’s condensed consolidated financial statements, which are prepared and presented in accordance with generally accepted accounting principles in the United States (GAAP), the Company uses the following non-GAAP financial measures in this press release: non-GAAP adjusted net loss and non-GAAP net loss per share. These non-GAAP financial measures exclude certain amounts or expenses from the corresponding financial measures determined in accordance with GAAP. Management believes this non-GAAP information is useful for investors, taken in conjunction with the Company’s GAAP financial statements, because it provides greater transparency and period-over- period comparability with respect to the Company’s operating performance and can enhance investors’ ability to identify operating trends in the Company’s business. Management uses these measures, among other factors, to assess and analyze operational results and trends and to make financial and operational decisions. Non-GAAP information is not prepared under a comprehensive set of accounting rules and should only be used to supplement an understanding of the Company’s operating results as reported under GAAP, not in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with GAAP. In addition, these non-GAAP financial measures are unlikely to be comparable with non-GAAP information provided by other companies. The determination of the amounts that are excluded from non-GAAP financial measures is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Reconciliations between these non-GAAP financial measures and the most comparable GAAP financial measures for the three and nine months ended September 30, 2024 and 2023 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

About the Avutometinib and Defactinib Combination

Avutometinib is a RAF/MEK clamp that induces inactive complexes of MEK with ARAF, BRAF and CRAF potentially creating a more complete and

durable anti-tumor response through maximal RAS/MAPK pathway inhibition. In contrast to currently available MEK-only inhibitors, avutometinib blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows avutometinib to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other MEK-only inhibitors.

Verastem Oncology is currently conducting clinical trials with avutometinib in RAS/MAPK driven tumors as part of its **R**af **A**nd **M**ek **P**rogram or RAMP. Verastem is currently enrolling patients and activating sites for RAMP 301 (NCT06072781) an international Phase 3 confirmatory trial evaluating the combination of avutometinib and defactinib, a selective FAK inhibitor, versus standard chemotherapy or hormonal therapy for the treatment of recurrent low-grade serous ovarian cancer (LGSOC). RAMP 201 (NCT04625270) is a Phase 2 registration-directed trial of avutometinib in combination with defactinib in patients with recurrent LGSOC and enrollment has been completed for the RAMP 201 trial.

Verastem has completed its submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for the investigational combination of avutometinib and defactinib in adults with recurrent KRAS mutant LGSOC who received at least one prior systemic therapy in October 2024, with a potential FDA decision mid-2025. The FDA granted Breakthrough Therapy Designation of the investigational combination of avutometinib and defactinib for the treatment of patients with recurrent LGSOC after one or more prior lines of therapy, including platinum-based chemotherapy. Avutometinib alone or in combination with defactinib was also granted Orphan Drug Designation by the FDA for the treatment of LGSOC.

Verastem Oncology has established a clinical collaboration with Amgen to evaluate LUMAKRAS™ (sotorasib) in combination with avutometinib and defactinib in both treatment naive and in patients who progressed on a G12C inhibitor as part of the RAMP 203 trial (NCT05074810). Verastem has received Fast Track Designation from the FDA for the triplet combination in April 2024. RAMP 205 (NCT05669482), a Phase 1b/2 clinical trial evaluating avutometinib and defactinib with gemcitabine/nab-paclitaxel in patients with front-line metastatic pancreatic cancer, is supported by the PanCAN Therapeutic Accelerator Award. FDA granted Orphan Drug Designation to avutometinib and defactinib combination for the treatment of pancreatic cancer.

About VS-7375/GFH375

VS-7375/GFH375 is a potential best-in-class, potent and selective oral KRAS G12D (ON/OFF) inhibitor, identified as the lead discovery program from the Verastem Oncology discovery and development collaboration with GenFleet Therapeutics. GenFleet's IND for VS-7375/GFH375 was approved in China in June 2024 and the Phase 1/2 trial in KRAS G12D-mutant solid tumors was subsequently initiated and the first patient was dosed in July 2024. The collaboration includes three discovery programs, the first being the KRAS G12D inhibitor, and provides Verastem Oncology with exclusive options to license three compounds selected for collaboration after successful completion of pre-determined milestones in Phase 1 trials. The licenses would give Verastem Oncology development and commercialization rights outside of the GenFleet territories of mainland China, Hong Kong, Macau, and Taiwan.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a late-stage development biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on RAS/MAPK-driven cancers, specifically novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including RAF/MEK inhibition and FAK inhibition. For more information, please visit www.verastem.com and follow us on LinkedIn.

Forward-Looking Statements

This press release includes forward-looking statements about, among other things, Verastem Oncology's programs and product candidates, strategy, future plans and prospects, including statements related to the expected timing for the FDA review of the rolling NDA submission for the avutometinib and defactinib combination in LGSOC, the structure of our planned and pending clinical trials, the potential clinical value of various of the Company's clinical trials, including the RAMP 201, 205 and 301 trials, the timing of commencing and completing trials, including topline data reports, interactions with regulators, the timeline and indications for clinical development, regulatory submissions and the potential for and timing of commercialization of product candidates and potential for additional development programs involving Verastem Oncology's lead compound, the expected outcome and benefits of our collaboration with GenFleet Therapeutics and the estimated addressable markets of our drug candidates. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "can," "promising" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and are subject to risks and uncertainties that could cause our actual results to differ materially from those expressed or implied in the forward-looking statements we make. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the success in the development and potential commercialization of our product candidates, including avutometinib in combination with other compounds, including defactinib, LUMAKRAS™ and others; the uncertainties inherent in research and development, such as negative or unexpected results of clinical trials, the occurrence or timing of applications for our product candidates that may be filed with regulatory authorities in any jurisdictions; whether and when regulatory authorities in any jurisdictions may approve any such applications that may be filed for our product candidates, and, if approved, whether our product candidates will be commercially successful in such jurisdictions; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding trial design, labeling and other matters that could affect the timing, availability or commercial potential of our product candidates; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that the market opportunities of our drug candidates are based on internal and third-party estimates which may prove to be incorrect; that third-party payors (including government agencies) may not reimburse; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected, which may delay our development programs, including delays in review by the FDA of our NDA submission in recurrent KRAS mutant LGSOC if enrollment in our confirmatory trial is not well underway at the time of submission, or that the FDA may require the Company to have completed enrollment or to enroll additional patients in the Company's ongoing RAMP-301 confirmatory Phase 3 clinical trial prior to the FDA taking action on our NDA seeking accelerated approval; risks associated with preliminary and interim data, which may not be representative of more mature data, including with respect to interim duration of therapy data; that our product candidates will cause adverse safety events and/or unexpected concerns may arise from additional data or analysis, or result in unmanageable safety profiles as compared to their levels of efficacy; that we may be unable to successfully validate, develop and obtain regulatory approval for companion diagnostic tests for our product candidates that require or would commercially benefit from such tests, or

experience significant delays in doing so; that the mature RAMP 201 data and associated discussions with the FDA may not support the scope of our NDA submission for the avutometinib and defactinib combination in LGSOC, including with respect to KRAS wild type LGSOC; that our product candidates may experience manufacturing or supply interruptions or failures; that any of our third party contract research organizations, contract manufacturing organizations, clinical sites, or contractors, among others, who we rely on fail to fully perform; that we face substantial competition, which may result in others developing or commercializing products before or more successfully than we do which could result in reduced market share or market potential for our product candidates; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned, including as a result of conducting additional studies or our decisions regarding execution of such commercialization; that we may not have sufficient cash to fund our contemplated operations, including certain of our product development programs; that we may not attract and retain high quality personnel; that we or Chugai Pharmaceutical Co., Ltd. will fail to fully perform under the avutometinib license agreement; that the total addressable and target markets for our product candidates might be smaller than we are presently estimating; that we or Secura Bio, Inc. (Secura) will fail to fully perform under the asset purchase agreement with Secura, including in relation to milestone payments; that we will not see a return on investment on the payments we have and may continue to make pursuant to the collaboration and option agreement with GenFleet Therapeutics (Shanghai), Inc. (GenFleet), or that GenFleet will fail to fully perform under the agreement; that we may not be able to establish new or expand on existing collaborations or partnerships, including with respect to in-licensing of our product candidates, on favorable terms, or at all; that we may be unable to obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates; and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients.

Other risks and uncertainties include those identified under the heading “Risk Factors” in the Company’s Annual Report on Form 10-K for the year ended December 31, 2023 as filed with the Securities and Exchange Commission (SEC) on March 14, 2024 and in any subsequent filings with the SEC, which are available at www.sec.gov. The forward-looking statements contained in this press release reflect Verastem Oncology’s views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

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Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	September 30, 2024	**December 31, 2023**
Cash, cash equivalents & short-term investments	$ 113,175	$ 137,129
Grant receivable	200	—
Prepaid expenses and other current assets	7,287	6,553
Property and equipment, net	39	37
Right-of-use asset, net	625	1,171
Restricted cash and other assets	5,052	4,828
Total assets	**$ 126,378**	**$ 149,718**
Current Liabilities	$ 37,374	$ 26,380
Long term debt	30,647	40,086
Lease liability, long-term	—	530

Preferred stock tranche liability	—	4,189
Warrant liability	26,138	—
Convertible preferred stock	21,159	21,159
Stockholders' equity	11,060	57,374
Total liabilities, convertible preferred stock and stockholders' equity	**$ 126,378**	**$ 149,718**

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Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2024	**2023**	**2024**	**2023**
Revenue				
Sale of COPIKTRA license and related assets	$ —	$ —	$ 10,000	$ —
Total revenue	—	—	10,000	—
Operating expenses:				
Research and development	24,754	13,946	60,523	38,854
Selling, general and administrative	12,276	7,363	32,843	22,091
Total operating expenses	37,030	21,309	93,366	60,945
Loss from operations	(37,030)	(21,309)	(83,366)	(60,945)
Other expense	(77)	(13)	(131)	(60)
Interest income	831	2,247	3,181	4,345
Interest expense	(1,148)	(1,129)	(3,416)	(3,019)
Change in fair value of preferred stock tranche liability	—	200	4,189	(320)
Change in fair value of warrant liability	13,457	—	13,457	—
Net loss	$ (23,967)	$ (20,004)	$ (66,086)	$ (59,999)
Net loss per share—basic and diluted	$ (0.60)	$ (0.75)	$ (2.11)	$ (2.93)[1]

Weighted average common shares outstanding used in computing:				
Net loss per share – basic and diluted	40,258	26,790	31,350	20,452[(1)]

(1) Amounts have been retroactively restated to reflect the 1-for-12 reverse stock split effected on May 31, 2023

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Reconciliation of GAAP to Non-GAAP Financial Information

(in thousands, except per share amounts)

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2024	**2023**	**2024**	**2023**
Net loss reconciliation				
Net loss (GAAP basis)	$ (23,967)	$ (20,004)	$ (66,086)	$ (59,999)
Adjust:				
Stock-based compensation expense	1,935	1,517	35,323	4,262
Non-cash interest, net	201	(371)	(212)	(295)
Change in fair value of preferred stock tranche liability	—	(200)	(4,189)	320
Change in fair value of warrant liability	(13,457)	—	(13,457)	—
Severance and Other	10	47	619	85
Adjusted net loss (non-GAAP basis)	$ (35,278)	$ (19,011)	$ (78,002)	$ (55,627)
Reconciliation of net loss per share				
Net loss per share – diluted (GAAP Basis)	(0.60)	(0.75)	(2.11)	(2.93)[(1)]
Adjust per diluted share:				
Stock-based compensation expense	0.05	0.06	0.17	0.21[(1)]
Non-cash interest, net	—	(0.01)	(0.01)	(0.02)[(1)]
Change in fair value of preferred stock tranche liability	—	(0.01)	(0.13)	0.02[(1)]

Change in fair value of warrant liability	(0.33)	—	(0.43)	—
Severance and Other	—	—	0.02	—
Adjusted net loss per share – diluted (non-GAAP basis)	$ (0.88)	$ (0.71)	$ (2.49)	$ (2.72)[1]
Weighted average common shares outstanding used in computing net loss per share—diluted	40,258	26,790	31,350	20,452[1]

(1) Amounts have been retroactively restated to reflect the 1-for-12 reverse stock split effected on May 31, 2023

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Source: Verastem Oncology